
Genome Editing to Correct Cystic Fibrosis Mutations in Airway Stem Cells

Grant Award Details

Genome Editing to Correct Cystic Fibrosis Mutations in Airway Stem Cells

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-09637

Project Objective: To develop a gene-edited, airway stem cell based therapeutic candidate for treating chronic sinusitis due to cystic fibrosis; AAV6-delivered CRISPR/Cas9 system will be developed to correct most common CF mutation (CFTR-Δ508), and/or replace entire CFTR gene, in airway stem cells to be transplanted into the nasal cavity.

Investigator:

Name:	Matthew Porteus
Institution:	Stanford University
Type:	PI

Disease Focus: Cystic Fibrosis, Respiratory Disorders

Human Stem Cell Use: Adult Stem Cell

Award Value: \$1,968,456

Status: Active

Grant Application Details

Application Title: Genome Editing to Correct Cystic Fibrosis Mutations in Airway Stem Cells

Public Abstract:**Research Objective**

Gene corrected autologous airway epithelial stem cells from patients with cystic fibrosis to be used as cell and gene based therapy for chronic sinus disease

Impact

The proposed studies would provide an innovative, readily applied primary stem cell based approach with gene correction to treat chronic sinusitis in CF, a debilitating airway disease.

Major Proposed Activities

- Identification of active CRISPR/Cas9 nucleases that can edit the human CFTR gene
- Develop a direct mutation correction genome editing approach for the deltaF508 mutation and a "Universal CFTR" correction system for the other CF causing mutations
- Identification of a genome editing delivery system for primary airway stem cells from CF patients
- Genome editing by homologous recombination in CF patient derived airway stem cells
- Test physiologic function of gene corrected CF airway stem cells when converted into organoids
- Xenotransplantation of gene corrected CF airway stem cells into NSG mice

Statement of Benefit to California:

Cystic fibrosis (CF) is one of the most common genetic diseases in California. There is no curative therapy for CF and CF patients spend a lifetime focused on mitigating the symptoms of their disease. Moreover, the costs of treating a single CF patient are enormous. Thus, the benefit to California if this proposal is successful is that it would improve the lives of its citizens (both patients and family members) while simultaneously decreasing the societal costs that this disease inflicts.

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